



now it's personal

**QUEST Program** 

Gil Sambrano, Ph.D.

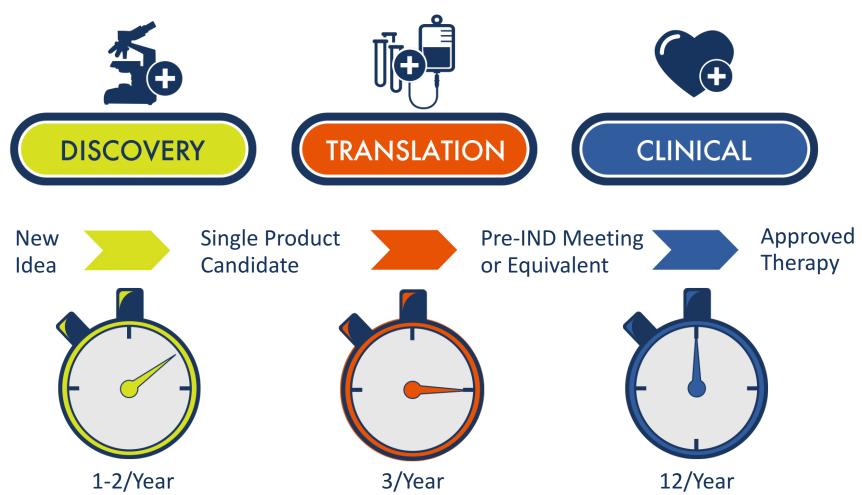
Director of Portfolio Development and Review





#### **Funding Opportunities**





Program Offerings Per Year



## CIRM Quest Discovery Program now it's personal

#### **Objective**

The Quest Program promotes the discovery of promising new stem cell-based technologies that will be ready for translational studies within two years to ultimately improve patient care.





#### Projects that propose a candidate:

- Therapeutic
- Diagnostic
- Medical device
- Tool

## What qualifies for Quest?



- Stem/progenitor cell therapy
- Reprogrammed cell therapy
- Small molecule or biologic that stimulates, recruits or targets human endogenous stem cells or cancer stem cells
- Device, diagnostic or tool that:
  - Uses stem/progenitor cells
  - Addresses a critical bottleneck in the stem cell therapy field

### **Review Criteria**



- ✓ Does the project hold the necessary significance and potential for impact?
- ✓ Is the rationale sound?
- ✓ Is the project well planned and designed?
- ✓ Is the project feasible?



## Scoring System

Score of "85-100"

Recommended for funding, if funds are available

Score of "1-84"

Not recommended for funding

Applications are scored by all scientific members of the GWG with no conflict.

The **median** of all individual GWG scores determines final score.





	Number of Apps	Total Applicant Request	Funds Available
Recommended for funding Score 85-100	11	\$21,057,289	\$25,832,311
Not recommended for funding Score 1-84	32		

For each award, the final award amount shall not exceed the amount approved by the ICOC Application Review Subcommittee and may be reduced contingent on CIRM's assessment of allowable costs and activities.



# Overview of Recommended Applications



TITLE: Preclinical development of an immune evasive islet cell replacement therapy for type 1 diabetes

**INDICATION:** Type 1 diabetes

**PRODUCT TYPE:** Cell therapy

**APPROACH:** Universal donor cell line



TITLE: Genome Editing of Sinusoidal Endothelial Stem Cells for Permanent Correction of Hemophilia A

INDICATION: Hemophilia A

PRODUCT TYPE: Gene therapy

APPROACH: In vivo correction of factor VIII gene



TITLE: Towards hepatocyte cell replacement therapy: developing a renewable source of human hepatocytes from pluripotent stem cells

**INDICATION:** Liver failure

**PRODUCT TYPE:** Cell therapy

**APPROACH:** Renewable hepatocytes from hPSCs



TITLE: Engineering Lifelong Cellular Immunity to HIV

**INDICATION:** HIV infection

PRODUCT TYPE: Gene-modified cell therapy

APPROACH: Modify blood stem cells to target

HIV



TITLE: iPS Glial Therapy for White Matter Stroke and Vascular Dementia

**INDICATION:** Vascular dementia

PRODUCT TYPE: Cell therapy

**APPROACH:** Glial enriched progenitors from

**iPSCs** 



TITLE: Stimulating endogenous muscle stem cells to counter muscle atrophy

**INDICATION:** Muscle atrophy

**PRODUCT TYPE: Small molecule** 

APPROACH: Drug to stimulate muscle stem cells



TITLE: Generation and in vitro profiling of neural stem cell lines to predict in vivo efficacy for chronic cervical spinal cord injury

**INDICATION:** Spinal cord injury

**PRODUCT TYPE:** Cell therapy

**APPROACH:** Human neural stem cell lines



TITLE: Silicon Nanopore Membrane encapsulated enriched-Beta Clusters for Type 1 Diabetes treatment

**INDICATION:** Type 1 diabetes

PRODUCT TYPE: Cell encapsulation device

**APPROACH:** Transplantation of beta cells in

device



TITLE: Identification and Generation of Long Term Repopulating Human Muscle Stem Cells from Human Pluripotent Stem Cells

**INDICATION:** Muscle disorders

PRODUCT TYPE: Cell therapy

**APPROACH:** Muscle stem cells from hPSCs



TITLE: Targeting Cancer Stem Cells in Hematologic Malignancies

INDICATION: Acute myeloid leukemia

PRODUCT TYPE: Monoclonal antibody

**APPROACH:** Antibody targets cancer stem cells



TITLE: Development of immune invisible beta cells as a cell therapy for type 1 diabetes through genetic modification of hESCs

**INDICATION:** Type 1 diabetes

**PRODUCT TYPE:** Cell therapy

**APPROACH:** Non-immunogenic beta cell

progenitors